IN THE UNITED STATES DISTRICT COURT
FOR THE DISTRICT OF DELAWARE

REGENXBIO INC. and THE TRUSTEES OF THE UNIVERSITY OF PENNSYLVANIA,	)	
Plaintiffs,	)	
v.	)	C.A. No. 20-1226 (RGA)
SAREPTA THERAPEUTICS, INC. and SAREPTA THERAPEUTICS THREE, LLC,	)	
Defendants.	)	

DEFENDANTS SAREPTA THERAPEUTICS, INC. AND SAREPTA THERAPEUTICS THREE, LLC'S OPENING BRIEF IN SUPPORT OF THEIR MOTION TO DISMISS PURSUANT TO FEDERAL RULE OF CIVIL PROCEDURE 12(b)(6)

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Defendants Sarepta Therapeutics, Inc. and Sarepta Therapeutics Three, LLC (collectively, "Sarepta") respectfully submit this Opening Brief in support of their Motion to Dismiss Pursuant to Federal Rule of Civil Procedure 12(b)(6).

I. NATURE AND STAGE OF PROCEEDINGS

On September 15, 2020, Plaintiffs RegenxBio Inc. and The Trustees of the University of Pennsylvania (collectively, "Plaintiffs") filed a Complaint against Sarepta alleging infringement under 35 U.S.C. §§ 271(a)-(c) of U.S. Patent No. 10,526,617 ("the '617 patent"). (D.I. 1). The Complaint alleges that Sarepta has infringed the '617 patent through its alleged use of certain "cultured host cells" to make a gene therapy product known as "SRP-9001." (*Id.* at ¶ 26). Notwithstanding the allegations of infringement, the Complaint acknowledges that Sarepta is currently only researching and developing SRP-9001 for the treatment of the rare disease Duchenne muscular dystrophy ("DMD"). (*Id.* at ¶ 35 ("SRP-9001 is currently in clinical development in the United States.")).[1]

Sarepta has moved to dismiss the Complaint for failure to state a claim for relief under Fed. R. Civ. P. 12(b)(6). For the reasons set forth below, Sarepta requests the Court to grant its motion and dismiss the Complaint in its entirety.

[1] In a footnote, Plaintiffs allege on "information and belief" that other Sarepta products infringe the '617 patent "for the same reasons" as SRP-9001. (*Id.* at ¶ 34 n.2). For the same reasons detailed for SRP-9001, any claims of patent infringement asserted against Sarepta's other products should be dismissed under Rule 12(b)(6) because those products are also only under research and development and not commercially available. (*See, e.g., id.*, Ex. L). Any such claims should also be dismissed because the Complaint includes no specific allegations against these other products. *See, e.g., DIFF Scale Operation Research, LLC v. MaxLinear, Inc.*, No. 19-2109-LPS-CJB, slip op. at 3, 2020 WL 2220031 at *1 (D. Del. May 7, 2020) (explaining that plaintiffs must "plead[] facts that plausibly indicate that Defendants' accused products practice each of the limitations asserted in the relevant claims").

II. SUMMARY OF ARGUMENT

The Court should dismiss Plaintiffs' Complaint pursuant to Fed. R. Civ. P. 12(b)(6) because Plaintiffs have failed to state a claim upon which relief may be granted. "To survive a motion to dismiss, a complaint must contain sufficient factual matter, accepted as true, to 'state a claim to relief that is plausible on its face.'" *See Ashcroft v. Iqbal*, 556 U.S. 662, 678 (2009). "A claim [only] has facial plausibility when the plaintiff pleads factual content that allows the court to draw the reasonable inference that the defendant is liable for the misconduct alleged." *Id.* (quoting *Bell Atl. Corp. v. Twombly*, 550 U.S. 544 (2007)). Here, the Complaint does not allow the Court to draw a reasonable inference that Sarepta is liable for patent infringement for at least three reasons.

1. *First,* Sarepta's research and development activities alleged in the Complaint fall squarely within the Safe Harbor protections of 35 U.S.C. § 271(e)(1). Section 271(e)(1) provides, *inter alia*, that it shall not be an act of infringement to use a patented invention "solely for uses reasonably related to the development and submission of information under a Federal law which regulates the manufacture, use, or sale of drugs. . . ." 35 U.S.C. § 271(e)(1). Plaintiffs allege that the "patented invention" referenced in the Safe Harbor is certain cultured host cells, and that Sarepta is allegedly infringing the '617 patent by using those cells to make SRP-9001. (D.I. 1 at ¶ 26). As the Complaint acknowledges, however, SRP-9001 is currently in clinical development and not yet commercially available. (*Id.* at ¶ 35; *see also, e.g., id.*, Ex. K at 9 (SRP-9001 belongs to a gene therapy class that "has already delivered a great deal of value, but in terms of potential, [is] still just an adolescent")). Thus, Plaintiffs' allegations as to Sarepta's use of their "patented invention" concern activities that are solely related to the development and future submission by Sarepta of a Biologics License Application ("BLA") to the FDA under the Federal Public Health

Service Act, 42 U.S.C. § 262 *et seq.* These activities are protected by the Safe Harbor and, as a matter of law, cannot form the basis of a patent infringement claim.

2. *Second,* the Complaint's allegation concerning Sarepta's License, Collaboration and Option Agreement with Roche Holdings AG ("the Roche Agreement") (*id*. at ¶ 35) does not transform the otherwise deficient Complaint into one stating a valid infringement claim. The Roche Agreement governs current research, development and regulatory efforts by Sarepta and Roche and only *possible future* commercialization of SRP-9001 if the companies are successful in obtaining the necessary regulatory approvals. (*See, e.g., id.*, Ex. H at 24 (defining "Commercialization" as activities "*following receipt of Regulatory Approval*") (emphasis added)). Further, even if the agreement itself is considered past or current commercial activity, it does not deprive Sarepta of the protections of the Safe Harbor. Pharmaceutical companies are not precluded *per se* from engaging in commercial activity related to a product under development. Instead, the Court must examine the Complaint's allegations and answer the question: Are the allegedly infringing activities – here, the alleged use of cultured host cells for the production of SRP-9001 – "solely for uses reasonably related to the development and submission of information" to the FDA? They are, and the Safe Harbor applies.

3. *Third*, the Complaint's allegation that "Sarepta has made and/or will make a commercial supply of SRP-9001" (D.I. 1 at ¶ 36) also does not transform the otherwise deficient Complaint into one that can survive a motion to dismiss. This allegation is based on Sarepta's most recent 10-K filing with the Securities and Exchange Commission ("SEC"). (*See id.* (citing to Sarepta's 10-K filing dated February 26, 2020 (Ex. H)). Sarepta's 10-K expressly states, however, that Sarepta is "clinically developing" SRP-9001 and that the referenced "commercial supply" is material for use in a *planned clinical trial* of SRP-9001, not a product intended for

sale. (*Id.*, Ex. H at 6 ("We plan to commence a ***trial*** evaluating SRP-9001 using commercial supply in the middle of 2020, pending regulatory feedback.") (emphasis added)). Sarepta intends to use "commercial supply" in its next clinical trial because the FDA encourages gene therapy companies to do so. (*See*, *e.g.*, FDA's Guidance for Industry on *Human Gene Therapy for Rare Diseases* ("FDA Gene Therapy Guidance," attached hereto as Exhibit 1) at 3 ("Sponsors are encouraged to consider, where possible, implementing manufacturing changes needed for commercial-scale production and demonstrating product comparability prior to the initiation of clinical trials intended to provide substantial evidence of effectiveness for a marketing application.")).[2] The FDA Gene Therapy Guidance, which applies to every prospective BLA applicant, does not deprive Sarepta of the protections of the Safe Harbor. Indeed, the Safe Harbor is intended to shield pharmaceutical companies from patent infringement claims precisely like those at issue here so that they may focus on developing lifesaving drugs, like SRP-9001.

III. STATEMENT OF FACTS

As the Complaint's exhibits explain, DMD is a rare, fatal neuromuscular genetic disease that affects children, with symptoms becoming increasingly noticeable in early childhood around ages three to five years old. (*See generally* D.I. 1, Ex. H at 6-12). DMD is caused by a defect in the gene that encodes a protein called "dystrophin," which is part of a group of proteins that work together to strengthen muscle fibers and protect them from injury as muscles contract and relax. (*Id.*). DMD results in muscle loss in the hips, thighs and pelvis, and makes it difficult for a child to walk or even stand. (*Id.*). Over time, the disease progresses to the arms, lower legs and trunk, and most children are in a wheelchair by the time they are 12 years old. (*Id.*). Due to an

[2] *See, e.g., Gustavson v. Wrigley Sales Co.*, 961 F.Supp.2d 1100, 1113 n.1 (N.D. Cal. 2013) (district court taking notice of FDA guidance document in context of motion to dismiss); *see also Schmidt v. Skolas*, 770 F.3d 241, 249 (3d Cir. 2014) (holding that court may take notice of matters of public record in context of motion to dismiss).

absence of dystrophin in the heart and lung muscles, children with DMD may also require assisted ventilation. (*Id.*). DMD affects approximately one in 3,500-5,000 males born worldwide.[3]

SRP-9001 is a potential gene therapy treatment that is being researched and developed by Sarepta for the treatment of DMD. (*Id.*). SRP-9001 includes a unique genetic construct that provides the muscle cells of a patient with a replacement dystrophin-type protein termed "micro-dystrophin." (*Id.*). This novel gene construct is delivered using a unique adeno-associated virus vector. (*Id.*). SRP-9001 has been described as the highest-potential gene therapy treatment for DMD and probably all of biotech. The full potential of SRP-9001, however, has yet to be realized because it is still undergoing clinical trials and not yet commercially available. (*Id.*). A BLA for SRP-9001 has not been submitted, and as such, SRP-9001 is not FDA approved. (*Id.*).

SRP-9001 is currently the subject of two ongoing clinical trials. (*Id.* at 7). The first trial (Study 101), which commenced in late 2017, is a Phase 1/2a trial of four individuals with DMD. (*Id.*). The second trial (Study 102), which commenced in late 2018, is a randomized, double-blind, placebo-controlled trial of 41 individuals. (*Id.*). The "commercial-supply" trial mentioned in the Complaint is a planned third clinical trial, with the goal of generating additional data on the safety and efficacy of SRP-9001 in support of a potential submission of a BLA with the FDA. (*Id.*). Per FDA guidelines, the finished dosage form of SRP-9001 to be used in this third clinical trial will be made using the same manufacturing process that would be used for "commercial supply" following FDA approval. (*Id.*). Sarepta has not yet started this third clinical trial. (*Id.*).

[3] The Court may also take judicial notice of these facts as disclosed on the National Institute of Health ("NIH") website at https://rarediseases.info.nih.gov/diseases/6291/duchenne-muscular-dystrophy. *See, e.g.*, *supra* note 2.

IV. ARGUMENT

The Court should dismiss the Complaint because Plaintiffs' allegations relating to Sarepta's ongoing and planned clinical trials of SRP-9001 to generate information for submission to the FDA are exempted from liability under the Safe Harbor. The Supreme Court set forth the basic standards that a plaintiff must satisfy to avoid a motion to dismiss in *Iqbal* and *Twombly*:

> To survive a motion to dismiss, a complaint must contain sufficient factual matter, accepted as true, to "state a claim to relief that is plausible on its face." A claim has facial plausibility when the plaintiff pleads factual content that allows the court to draw the reasonable inference that the defendant is liable for the misconduct alleged. The plausibility standard is not akin to a "probability requirement," but it asks for more than a sheer possibility that a defendant has acted unlawfully. Where a complaint pleads facts that are "merely consistent with" a defendant's liability, it "stops short of the line between possibility and plausibility of 'entitlement to relief.'"

Iqbal, 556 U.S. at 678 (quoting *Twombly*, 550 U.S. at 556-57, 570).

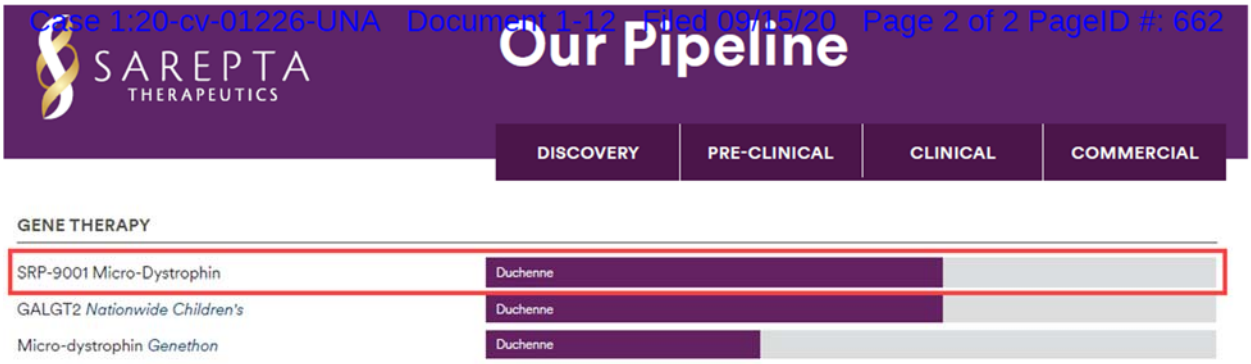
Following *Iqbal* and *Twombly*, the Third Circuit and Courts in this district have further explained that "a complaint must do more than simply provide 'labels and conclusions' or 'a formulaic recitation of the elements of a cause of action.'" *NNCrystal US Corp. v. Nanosys, Inc.*, 2020 WL 616307 at *2 (D. Del. 2020) (citing *Davis v. Abington Mem'l Hosp.*, 765 F.3d 236, 241 (3d Cir. 2014)). "[The Court is] not required to credit bald assertions or legal conclusions improperly alleged in the complaint." *Id.* (citing *In re Rockefeller Ctr. Props., Inc. Sec. Litig.*, 311 F.3d 198, 216 (3d Cir. 2002)); *see also DiStefano Patent Tr. III, LLC v. LinkedIn Corp.*, 346 F. Supp. 3d 616, 620 (D. Del. 2018) (holding that a court need not accept bald assertions, unsupported conclusions, unwarranted inferences or self-evidently false allegations) (citations omitted).

Here, Plaintiffs have not pled a cause of action that allows the Court to draw a reasonable inference that Sarepta is liable for patent infringement. The Safe Harbor protects Sarepta's manufacture and use of SRP-9001, and Plaintiffs' sole allegations attempting to support commercial activities – the Roche Agreement and the manufacture of a "commercial supply" for use in a planned clinical trial – do not deprive Sarepta of those protections.

A. The Safe Harbor Protects Sarepta's Research and Development Activities for SRP-9001

Plaintiffs' infringement allegations with respect to Sarepta's research and development of SRP-9001 fall squarely within the Safe Harbor. The Complaint alleges that SRP-9001 is currently in clinical development. (D.I. 1 at ¶ 35 ("On information and belief, SRP-9001 is currently in clinical development in the United States.")). In addition, the Complaint alleges that SRP-9001 is made using a process that includes making and using cultured host cells that allegedly infringe the '617 patent. (*See, e.g.*, D.I. 1 at ¶¶ 1, 18, 26-32, 36-37). Sarepta's alleged use of cultured host cells to produce SRP-9001, however, is in support of an Investigational New Drug Application (IND) seeking FDA authorization to perform clinical trials and for any BLA that Sarepta may submit seeking FDA approval to market SRP-9001 if those clinical trials are successful. FDA regulations require submission of this type of manufacturing information to the FDA with an applicant's IND or BLA. *See* 21 C.F.R. § 312.23(a)(7) (IND requires manufacturing and other information for the biologic product); 21 C.F.R. § 601.2 (BLA requires, *inter alia*, a "full description of manufacturing methods" for the biologic product). Because Sarepta's alleged use of cultured host cells to manufacture SRP-9001 would generate information for submission to the FDA, that activity qualifies for Safe Harbor protection under 35 U.S.C. § 271(e)(1).

The Complaint's exhibits similarly confirm that SRP-9001 is currently involved only in clinical trials and that no potentially infringing commercial activity has taken place. For example, Exhibit L is a graphic of Sarepta's product pipeline and shows that SRP-9001 is currently in clinical trials:



(D.I. 1, Ex. L).

Similarly, Exhibits E through K repeatedly explain that SRP-9001 is only in clinical trials. For example, the exhibits state:

- "With that goal in mind, we are excited to support ***clinical development*** for Nationwide's gene therapy program with the goal to help all boys with DMD." (*Id.*, Ex. E, January 10, 2017 Sarepta Press Release);

- "In the fourth quarter of 2017, an ***investigational new drug ("IND")*** application for the micro-dystrophin gene therapy program was cleared by the FDA, and a ***Phase 1/2a clinical trial*** in individuals with DMD was initiated." (*Id.*, Ex. H, Sarepta's 10-K filing dated February 26, 2020, at 9);

- "In the fourth quarter of 2018, we commenced a ***randomized, double-blind, placebo-controlled trial of SRP-9001*** with the goal to establish the functional benefits of micro-dystrophin expressions. We have dosed all 41 participants in that trial and have begun dosing participants in the crossover phase of the study." (*Id.*, Ex. H, Sarepta's 10-K filing dated February 26, 2020, at 9); and

- "We plan to commence a ***trial*** evaluating SRP-9001 using commercial supply in the middle of 2020, ***pending regulatory feedback***." (*Id.*, Ex. H, Sarepta's 10-K filing dated February 26, 2020, at 9).

The Complaint cites these exhibits but omits the relevant portions noted above. These documents confirm that Sarepta's activities with SRP-9001 are limited to the generation and submission of clinical trial data to the FDA and, as such, are protected by the Safe Harbor. Considering the Complaint's allegations and exhibits, the Court cannot reasonably infer that Sarepta is engaged in any activity with SRP-9001 that is not protected by the Safe Harbor. *See, e.g.*, *Classen Immunotherapies, Inc. v. Shionogi, Inc.*, 993 F. Supp. 2d 569, 575-78 (D. Md. 2014) (granting motion to dismiss patent infringement complaint based on application of the Safe Harbor), aff'd, 586 F. App'x 585 (Fed. Cir. 2014).[4]

B. The Roche Agreement Does Not Deprive Sarepta of the Protections of the Safe Harbor

Recognizing that Sarepta's activities to date with SRP-9001 are protected by the Safe Harbor, Plaintiffs attempt to avoid dismissal by including an allegation in their Complaint that characterizes the Roche Agreement as commercial in nature. (*See*, *e.g.*, D.I. 1 at ¶ 35 ("On information and belief, Sarepta Three has entered into an agreement with [Roche] to develop and commercialize SRP-9001 outside the United States.")). The documents cited in the Complaint, however, demonstrate that the Roche Agreement addresses only *future possible* commercialization of SRP-9001, not current commercial activities. (*See, e.g.*, *id.*, Ex. H at 24 (defining "Commercialization" as certain activities that occur "following receipt of Regulatory Approval")). Further, even if the agreement could be considered past or current commercial activity, it does not deprive Sarepta of the Safe Harbor protections. Commercial activities related to a product under research and development – including the negotiation and execution of

[4] Plaintiffs state that although SRP-9001 requires FDA approval for marketing, "the cultured host cells claimed in the '617 Patent, and used by Sarepta to produce SRP-9001, do not." (D.I. 1 at ¶ 34). The relevant question, however, is not whether the cultured host cells require FDA approval, but whether the allegedly infringing activities are solely for purposes reasonably related to the development and submission of information to the FDA. *See* 35 U.S.C. § 271(e)(1). Because they are, the Safe Harbor applies.

agreements like the Roche Agreement – do not deprive a pharmaceutical company of the protections of the Safe Harbor *per se*. Rather, a court must examine the specific allegations of infringement in the complaint and determine whether the alleged use of the "patented invention" is for anything other than the development and submission of information to the FDA. If it is not, the complaint should be dismissed. *See, e.g.*, *Abtox, Inc. v. Exitron Corp.*, 122 F.3d 1019 (Fed. Cir. 1997); *Telectronics Pacing Sys., Inc. v. Ventritex, Inc.*, 982 F.2d 1520 (Fed. Cir. 1992); *Med. Diagnostic Labs., L.L.C. v. Protagonist Therapeutics, Inc.*, 298 F. Supp. 3d 1241 (N.D. Cal. 2018).

For example, in *Abtox*, Abtox alleged that Exitron and MDT infringed its patent covering sterilization devices by conducting tests to collect data for FDA submission. *Abtox*, 122 F.3d at 1027. Abtox argued that the testing was outside of the Safe Harbor because "the actual purpose of these tests was not to secure FDA approval, but was intended, *inter alia*, to promote the plasma sterilizer and other equipment to potential customers and induce MDT to purchase the rights to the device, which it [ultimately] did." *Id.* The Federal Circuit disagreed, holding that the Safe Harbor protected the tests because they were reasonably related to seeking FDA approval, and the company's intent or alternative uses were "irrelevant to its qualification to invoke the section 271(e)(1) shield." *Id.* at 1030.

Likewise, in *Telectronics*, Telectronics sued Ventritex for infringing its patents covering an implantable defibrillator. *Telectronics*, 982 F.2d at 1521. Telectronics alleged that while Ventritex was conducting its clinical trials, Ventritex had engaged in commercial activities that were not exempt under the Safe Harbor, including reporting clinical trial progress to investors, and describing clinical trial results in a private fund-raising memorandum. *Id.* at 1523. The court rejected the argument, holding that neither the plain meaning of § 271(e)(1) nor the legislative

history supported a finding that activities protected under the Safe Harbor lose protection when the sponsoring company is conducting commercial activities in preparation for the future commercial launch of its product. *Id.* at 1523-24. The court explained that "[i]t would strain credulity to imagine that Congress was indifferent to the economics of developing and marketing drugs and medical devices when it enacted [the Safe Harbor]." *Id.* at 1525.

Finally, *Med. Diagnostic Labs* is strikingly similar to the allegations here. MDL sued Protagonist for infringing its patents covering IL–23 receptor inhibitor technology based on Protagonist's research and development of a drug candidate ("PTG-200"). *Med. Diagnostic Labs*, 298 F. Supp. 3d at 1246. MDL argued that Protagonist's activities were not covered by the Safe Harbor because Protagonist had entered into a collaboration agreement with Janssen Biotech to "develop, manufacture, and commercialize PTG-200 worldwide for the treatment of Crohn's disease and ulcerative colitis." *Id*. Under the agreement, Janssen made an initial payment of $50 million to fund the research for PTG-200 and had the option to maintain its rights under the agreement in exchange for future milestone payments up $940 million. *Id.* MDL argued that the Janssen agreement was evidence of commercial activity that was not protected by the Safe Harbor. *Id.* at 1246-47. At the time of the litigation, "PTG-200 [was] still in pre-clinical trials and no regulatory approval [had] been obtained or even sought." *Id.* at 1246.

Relying on *Abtox* and *Telectronics*, the court held that the Janssen agreement did not deprive Protagonist of the Safe Harbor protections. *Id*. at 1251. According to the court, the initial payment under the agreement was "clearly reasonably related to the development and submission of information for the FDA approval process," and "[w]hether Protagonist [was] ultimately motivated to use that research (or the money) to promote or commercialize the drug [was] immaterial under *Abtox*." *Id.* The court held that the agreement's future payment provisions also

did not take Protagonist outside the Safe Harbor because "the agreement [was] explicitly structured around the stages of FDA approval." *Id.*

The same is true here. Like the Janssen agreement in *Med. Diagnostic Labs*, the Roche Agreement is a collaboration and option agreement between Sarepta and Roche related to the development of SRP-9001. (D.I. 1, Ex. H at 14 (Roche Agreement titled "License, Collaboration, and Option Agreement")). Like the Janssen agreement, the Roche Agreement provides for an initial upfront payment to Sarepta "for the rights granted by Sarepta and for prepaid funding for Development activities" and future milestone payments based on the potential achievement of various regulatory milestone events. (*Id.* at 81). And like PTG-200, SPR-9001 has not been approved by the FDA and is not yet the subject of a BLA. (*See supra* Section IV(A)). This Court should therefore hold that the Roche Agreement does not take Sarepta outside of the Safe Harbor. As the *Med. Diagnostic Labs* court succinctly explained:

> In crafting the safe harbor, 'Congress understood that in the real world of high-tech medicine, at least, it is 'business purposes' that inspire the kinds of infringing activities that the exemption clearly covers.' Against this backdrop, 'it would strain credulity to imagine that Congress was indifferent to the economics of developing and marketing drugs and medical devices when it enacted § 271(e)(1).'

Id. at 1252 (quoting *Intermedics, Inc. v. Ventritex, Inc.*, 775 F. Supp. 1269, 1279 (N.D. Cal. 1991)) and *Telectronics*, 982 F.2d at 1525; *see also Telectronics*, 982 F.2d 1525 n.5 (Federal Circuit expressly endorsing the *Intermedics* decision).

C. Plaintiffs' Allegation of a "Commercial Supply" of SRP-9001 for Use in a Planned Clinical Trial Does Not Deprive Sarepta of the Safe Harbor Protections

Plaintiffs also attempt to avoid the Safe Harbor by including in ¶ 36 of their Complaint a conclusory allegation that "[u]pon information and belief, Sarepta has made and/or will make a commercial supply of SRP-9001." (D.I. 1 at ¶ 36). In support of this allegation, Plaintiffs cite to

Sarepta's most recent 10-K filing where Sarepta states that it "plan[s] to commence a trial evaluating SRP-9001 using commercial supply in the middle of 2020, pending regulatory feedback." (*Id.*, Ex. H at 4). This statement does not deprive Sarepta of Safe Harbor protections.

The phrase "commercial supply" in Sarepta's 10-K is referring to material for use in Sarepta's planned clinical trial pursuant to FDA guidance, not any product for commercial sale or other non-FDA use. The FDA encourages prospective BLA applicants like Sarepta to correlate the finished drug product used in clinical trials with the drug product that would be marketed and sold if ultimately FDA approved. In its Gene Therapy Guidance, the FDA explains that gene therapy companies must submit data that "demonstrat[e] process control to ensure a consistent product." (Ex. 1, FDA Gene Therapy Guidance, at 2). Because this can be particularly challenging in the context of gene therapy products, the FDA encourages prospective BLA applicants like Sarepta to "implement[] manufacturing changes needed for commercial-scale production and demonstrate[] product comparability prior to the initiation of clinical trials intended to provide substantial evidence of effectiveness for a marketing application." (*Id.* at 2-3). This concept is also explained in the Piper Jaffray slide presentation included as Exhibit K to the Complaint. Although Plaintiffs attach only a portion of the presentation, the full presentation is attached hereto as Exhibit 2.[5] Slide 35 – omitted by Plaintiffs – also explains that the FDA Gene Therapy Guidance encourages the use of "commercial material" in clinical trials:

[5] The Court may consider the complete document. *See, e.g.*, *Perrigo Co. v. Int'l Vitamin Corp.*, 2018 WL 4290387, at *2 (D. Del. Sept. 7, 2018) (holding that, in the context of a Rule 12(b)(6) motion, "it is permissible to consider the full text of documents partially quoted in complaint").

(Ex. 2 at 35).

Further, the cited statement from Sarepta's 10-K explains that Sarepta is only *planning* a "trial evaluating SRP-9001 using commercial supply," not presently conducting the trial. (D.I. 1, Ex. H at 6). In its 10-K, Sarepta unequivocally indicates that regulatory feedback is a prerequisite to the commencement of the planned clinical trial. Plaintiffs have cited no public announcement indicating that Sarepta received regulatory feedback, and any suggestion that Sarepta conducted the clinical trial or manufactured a "commercial supply" without regulatory feedback is a bald assertion that the Court should not accept as true. *See NNCrystal US Corp.*, 2020 WL 616307 at *2 ("[The Court is] not required to credit bald assertions or legal conclusions improperly alleged in the complaint.") (citing *In re Rockefeller Ctr. Props., Inc. Sec. Litig.*, 311 F.3d at 216)).

D. The Court Should Dismiss the Complaint With Prejudice Because Amendment Would Be Futile

The Court should dismiss the Complaint with prejudice rather than allow amendment. Leave to amend a complaint may be denied where amendment would be futile. Fed. R. Civ. P. 15(a). Amendment is "futile" where the complaint, as amended, would fail to state a claim on

which relief could be granted. *See Grayson v. Mayview State Hosp.*, 293 F.3d 103, 113 (3d Cir. 2002).

Here, there are no facts with which Plaintiffs could amend their Complaint that would provide a reasonable basis for asserting that Sarepta's conduct in the clinical development of SRP-9001 amounts to patent infringement. Given the statutory protections of the Safe Harbor, even if Plaintiffs amended their Complaint to assert declaratory judgment jurisdiction under 28 U.S.C. §2201, the Complaint would be subject to dismissal pursuant to Rule 12(b)(1). *Juno Therapeutics, Inc. v. Kite Pharma, Inc.*, No. 16-1243-RGA, 2017 WL 2559735 (D. Del. June 13, 2017); *Clarus Therapeutics, Inc. v. Lipocine, Inc.*, No. 15–1004–RGA–MPT, 2016 WL 5868065 (D. Del. Oct. 6, 2016).

For example, in *Juno*, plaintiffs sought a declaratory judgment that defendant's therapy would infringe their patent in the near future. *Juno*, 2017 WL 2559735 at *1. Plaintiffs did not dispute that defendant's activities were related to the development of information for submission to the FDA. *Id.* at *2. This Court held that plaintiffs' claims of patent infringement failed under the immediacy prong of declaratory judgment jurisdiction because there was no certainty that the FDA would approve defendant's BLA. *Id.* at *3. The Court also explained that even if there were a basis for declaratory judgment jurisdiction, the Court would decline to exercise such jurisdiction because it "would conflict with the purpose of the Safe Harbor Provision of the Patent Act." *Id*. The Court emphasized that "[t]he intent of the Safe Harbor is to provide drug manufacturers with protection for activities related to seeking FDA approval." *Id.; see also Clarus*, 2016 WL 5868065 at *4 (declining to exercise declaratory judgment jurisdiction "where Defendant is not currently infringing, has not engaged in any product marketing, and has not solicited orders, would be to allow Plaintiff to circumvent the Safe Harbor Provision.").

Like *Juno*, Sarepta is not engaged in any infringing activity, but only research and development that is protected by the Safe Harbor. Sarepta is not marketing or selling SRP-9001. Indeed, Sarepta has neither submitted a BLA to the FDA for SRP-9001 nor even commenced its planned clinical trial using commercial process material necessary to generate the data and information to support a future BLA submission for SRP-9001. (*See* D.I. 1, Ex. H at 6).

V. CONCLUSION

Even taking the allegations in Plaintiffs' Complaint as true, the alleged infringing conduct falls within the statutory Safe Harbor exemption. For the above reasons, Sarepta respectfully requests that the Court dismiss the Complaint with prejudice pursuant to Fed. R. Civ. P. 12(b)(6).[6]

[6] In the alternative, the Court should dismiss the Complaint pursuant to Rule Fed. R. Civ. P. 12(b)(1) because the Court lacks jurisdiction when, as here, the complaint is "absolutely devoid of merit" or "obviously frivolous." *Dubose v. Walsh*, No. 07–045–SLR–LPS, 2008 WL 4426090 at *3 (D. Del. Sept. 29, 2008) ("Claims that are 'absolutely devoid of merit' and 'obviously frivolous' will divest a district court of jurisdiction.") (quoting *Hagans v. Lavine*, 415 U.S. 528, 536-37 (1989)).

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November 4, 2020

CERTIFICATE OF SERVICE

I hereby certify that on November 4, 2020, I caused the foregoing to be electronically filed with the Clerk of the Court using CM/ECF, which will send notification of such filing to all registered participants.

I further certify that I caused copies of the foregoing document to be served on November 4, 2020, upon the following in the manner indicated:

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